



# 10

## SEQUENCE LISTING

<110> Mansfield, Gary S.  
Mitchell, Lloyd G.  
Garcia-Blanco, Mariano A.  
Walsh, Christopher E.  
Chao, Hengjun

<120> METHODS AND COMPOSITIONS FOR USE IN  
SPLICEOSOME MEDIATED RNA TRANS-SPlicing

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<140> 09/838,858  
<141> 2001-04-20

<150> 09/756,096  
<151> 2001-02-08

<150> 09/158,863  
<151> 1998-09-23

<150> 09/133,717  
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<150> 09/087,233  
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<150> 08/766,354  
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<220>  
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<400> 37  
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<210> 38  
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<210> 39

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<212> DNA  
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<212> DNA  
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<210> 53  
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gonadotropin gene 6 sequences and Corynebacterium  
diphtheriae toxin A sequence

<400> 53  
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24

<210> 54  
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<221> unsure  
<222> (57)...(70)  
<223> A, C, G or U

<221> misc feature  
<222> (57)...(70)  
<223> Loop comprising a combination of 14 nucleotides

<400> 54  
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nnnnnnnnnnn aucguuaacu aauaaacuac uaacugggug aacuucuguu uuuuucucga 120  
gcugcag 127

<210> 55  
<211> 127

<212> RNA

<213> Artificial Sequence

<220>

<223> PTM intramolecular base paired stem

<221> unsure

<222> (57)...(70)

<223> A, C, G or U

<221> misc\_feature

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<223> Loop comprising a combination of 14 nucleotides

<400> 55

gcuagccugg gacaaggaca cugcuucacc cgguuaguag accacagccc ugagccnnnn 60  
nnnnnnnnnnn aucguuaacu aauaaacuac uaacugggug aacuucugua uuauucucga 120  
gcugcag 127

<210> 56

<211> 127

<212> RNA

<213> Artificial Sequence

<220>

<223> PTM intramolecular base paired stem

<221> unsure

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<223> A, C, G or U

<221> misc\_feature

<222> (57)...(70)

<223> Loop comprising a combination of 14 nucleotides

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gcugcag 127

<210> 57

<211> 132

<212> DNA

<213> Artificial Sequence

<220>

<223> Trans-spliced product containing human chorionic  
gonadotropin gene 6 sequences and Corynebacterium  
diphtheriae diphtheria toxin A sequences

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tccattcaaa aa 132

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<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

<400> 58  
gaattcggta ccatgggg 18

<210> 59  
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<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

<400> 59  
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<220>  
<223> Artificial Sequence derived from Escherichia coli  
lacZ gene

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<210> 61  
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<212> DNA  
<213> Artificial Sequence

<220>  
<223> Trans-spliced product containing Escherichia coli  
lacZ gene sequences and human chorionic  
gonadotropin gene 6 exon 2 sequences

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<210> 62  
<211> 286  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Trans-spliced product containing Escherichia coli  
lacZ gene sequences

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gtAACAGTCT tggcggttc gctaaatact ggcaggcggt tcgtcagtat ccccgttac 120  
aggcggttt cgtctaataa tgggactggg tggatcagtc gctgattaaa tatgtatgaaa 180  
acggcaacc cgtggcggc ttacggcggt gattttggcg atacgcccggaa cgatcgccag 240  
ttctgtatga acggctcggt ctttgccgac cgacgcccgc atccag 286

<210> 63  
<211> 196  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Trans-spliced product containing Escherichia coli  
lacZ gene sequences

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gtAACAGTCT tggcggttc gctaaatact ggcaggcggt tcgtcagtat ccccgttac 120  
aggcggtct gctgttgctg ctgctgagca tgggccccggac atgggcattcc aaggagccac 180  
ttcggccacg gtggcc 196

<210> 64  
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<212> DNA  
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<220>  
<223> Trans-spliced product containing cystic fibrosis  
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His-tag sequence

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tgatgattat gggagaactg gagccttcag aggtaaaaat taagcacagt ggaagaattt 180  
cattctgttc tcagtttcc tggattatgc ctggcaccat taaagaaaaat atcatctttg 240

gcggccgcca ctgtgctgga tatctgcaga attccaccac actggactag tggatccgag 300  
ctcggtacca aggttaagtt taaaccgctg atcagcctcg actgtgcctt ctagttgcca 360  
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<220>  
<223> Splice junction sequence

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<210> 66  
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<213> Artificial Sequence

<220>  
<223> C terminal residus from glutathione-S- tranferase

<400> 66  
Asp Tyr Lys Asp Asp Asp Lys  
1 5

<210> 67  
<211> 15  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Artificial sequence comprising sequences derived  
from Esherichia coli lacZ gene sequences

<400> 67  
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15

<210> 68  
<211> 37  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Artificial sequence comprising sequences derived  
from Esherichia coli lacZ gene sequences

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<210> 69  
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<212> DNA  
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<220>  
<223> Binding domain of PTM

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<213> Artificial Sequence

<220>  
<223> Spacer sequence of PTM

<400> 70  
aacattatta taacgttgct cgaa 24

<210> 71  
<211> 47  
<212> DNA  
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<220>  
<223> Branch point, pyrimidine tract and acceptor splice site of PTM

<400> 71  
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<210> 72  
<211> 70  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Donor site and spacer sequence of PTM

<400> 72  
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gatccacccgg 70

<210> 73  
<211> 260  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Binding domain of spacer sequence

<400> 73  
tcaaaaagtt ttcacataat ttcttacctc ttcttgaatt catgcttga tgacgcttct 60  
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ctggaaaact gataacacaa taaaattctt ccactgtgct taaaaaaacc ctcttgaatt 180  
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aactcattat caaatcacgc 260

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<220>  
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<400> 74  
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<210> 75  
<211> 23  
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<220>  
<223> Oligonucleotide

<400> 75  
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<210> 76  
<211> 36  
<212> DNA  
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<220>  
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<400> 76  
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<210> 77

<211> 33  
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<210> 78  
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<220>  
<223> Oligonucleotide primer

<400> 78  
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<210> 79  
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<220>  
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<400> 79  
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<210> 80  
<211> 37  
<212> DNA  
<213> Artificial Sequence

<220>  
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<210> 81  
<211> 23  
<212> DNA  
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<220>  
<223> Binding domain of PTM molecule

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23

<210> 82  
<211> 22  
<212> DNA  
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<220>  
<223> Oligonucleotide primer

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22

<210> 83  
<211> 21  
<212> DNA  
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<220>  
<223> Oligonucleotide primer

<400> 83  
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<210> 84  
<211> 22  
<212> DNA  
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<220>  
<223> Oligonucleotide primer

<400> 84  
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22

<210> 85  
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<213> Artificial Sequence

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site.

<221> misc\_feature  
<222> (7)...(30)  
<223> Spacer sequence, see SEQ ID NO: 70

<221> unsure

<222> (7)...(30)

<223> A, C, G or T

<400> 85

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52

<210> 86

<211> 71

<212> DNA

<213> Artificial Sequence

<220>

<223> Oligonucleotide

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71

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<212> DNA

<213> Artificial Sequence

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<400> 87

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66

acgccc

<210> 88

<211> 192

<212> DNA

<213> Artificial Sequence

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<223> PTM sequence

<400> 88

acgagcttgc tcatgatgat catggcgag ttagaaccaa gtgaaggcaa gatcaaacat 60

120

tccggccgca tcagctttg cagccaattc agttggatca tgcccggtac catcaaggag

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192

cagttggagg ag

<210> 89

<211> 25

<212> DNA

<213> Artificial Sequence

<220>  
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<400> 89 25  
gagcaggcaa gacgagcttg ctcac

<210> 90  
<211> 28  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Oligonucleotide

<400> 90 28  
gagaacataa tcttcggcgt cagttacg

<210> 91  
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<220>  
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<400> 91 30  
gtcagttgga ggaggacatc tccaaatgg

<210> 92  
<211> 192  
<212> DNA  
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<220>  
<223> Oligonucleotide

<400> 92  
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tccggccgca tcagctttt cagccattc agttggatca tgcccggtac catcaaggag 120  
aacataatct tcggcgatc ttacgacgag taccgctatc gctcggtat taaggcctgt 180  
cagttggagg ag 192

<210> 93  
<211> 27  
<212> DNA  
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<220>  
<223> PTM sequences

<400> 93  
aaatatcatt ggtgtttctt atgatga 27

<210> 94  
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<213> Artificial Sequence

<220>  
<223> Oligonucleotide

<400> 94  
ccaactagaa gaggacatct ccaagttgc 30

<210> 95  
<211> 30  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Oligonucleotide

<400> 95  
atgatcatgg gcgaggttaga accaagttag 30

<210> 96  
<211> 27  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Oligonucleotide

<400> 96  
aaaatatcat ctgggtgtt tcctatg 27

<210> 97  
<211> 27  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Oligonucleotide

<400> 97  
ccaactagaa gaggacatct ccaagtt 27

<210> 98  
<211> 21  
<212> DNA

<213> Artificial Sequence

<220>

<223> 5' splice site

<400> (98

cgtttacagg taagtggatc c

21

<210> 99

<211> 27

<212> DNA

<213> Artificial Sequence

<220>

<223> 3' splice site

<400> 99

ctgcagggcg gcttcgtcta ataatgg

27

<210> 100

<211> 65

<212> DNA

<213> Artificial Sequence

<220>

<223> Sequence from trans-splicing domain

<221> unsure

<222> (7)...(18)

<223> A, C, G or T

<400> 100

gctagcnnnc cgcggnnta ctaactggta cctcttcttt ttttttgat atcctgcagg 60  
gcggc 65

<210> 101

<211> 1584

<212> DNA

<213> Artificial Sequence

<220>

<223> CFTR PTM

<400> 101

atgcagaggt cgcctctgga aaaggccagc gttgtctcca aactttttt cagctggacc 60  
agaccaattt tgaggaaagg atacagacag cgcctgaaat tgcagacat ataccaaatc 120  
ccttctgttg attctgctga caatctatct gaaaaattgg aaagagaatg ggatagagag 180  
ctggcttcaa agaaaaatcc taaactcatt aatgcccttc ggcgatgtt tttctggaga 240  
tttatgttct atggaatctt tttatattta gggaaagtca ccaaagcagt acagcctctc 300  
ttactggaa gaatcatagc ttcctatgac ccggataaca aggaggaacg ctctatcg 360

atttatctag gcataggctt atgccttctc tttattgtga ggacactgct cctaçaccca 420  
gccattttg gccttcatca cattggaatg cagatgagaa tagctatgtt tagtttgatt 480  
tataagaaga cttaaagct gtcaagccgt gttctagata aaataagtat tggacaactt 540  
gttagtctcc ttccaacaa cctgaacaaa tttgatgaag gacttgcatt ggcacattc 600  
gtgtggatcg ctcccttgca agtggcactc ctcatgggc taatctggga gttgttacag 660  
gcgtctgcct tctgtggact tggtttctg atagtccttgc cctttttca ggctgggcta 720  
gggagaatga ttagtgaagta cagagatcg agagctggga agatcagtga aagacttgc 780  
attacctcg aaatgatcga gaacatccaa tctgttaagg catactgctg ggaägaagca 840  
atggaaaaaa tgattgaaaaa cttaaagacaa acagaactga aactgactcg gaaggcagcc 900  
tatgtgagat acttcaatag ctccagcttc ttcttctc tag ggttctttgt ggtgtttta 960  
tctgtgcttc cctatgcact aatcaaagga atcatcctcc gaaaaatatt caccaccatc 1020  
tcattctgca ttgttctgcg catggcggc actcggcaat ttccctggc tgtacaaaca 1080  
tggatgact ctcttggagc aataaacaaa atacaggatt tcttacaaa gcaagaatatt 1140  
aagacattgg aatataactt aacgactaca gaagtagtga tggagaatgt aacagccttc 1200  
tgggaggagg gattttggaa attatttgag aagcaaaac aaaacaataa caatagaaaa 1260  
acttctaattg gtgtgacag cctcttcttc agtaattttt cacttcttgg tactcctgtc 1320  
ctgaaagata ttaatttcaa gatagaaaaga ggacagttgt tggcggttgc tggatccact 1380  
ggagcaggca agacgagctt gctcatgatg atcatggcgt agttagaacc aagtgaaggc 1440  
aagatcaaac attccggccg catcagctt tgcagccaaat ttagttggat catgcccgg 1500  
accatcaagg agaacataat ctccggcgtc agttacgacg agtaccgcta tcgctcggtg 1560  
attaaggcct gtcagttgga ggag 1584

<210> 102

<211> 323

<212> DNA

<213> Artificial Sequence

<220>

<223> Trans-splicing domain of CFTR PTM

<400> 102

gtaagatatc accgatatagt gtctaaccctg attcgggcct tcgatacgct aagatccacc 60  
ggtaaaaaag ttttcacata atttcttacc ttttcttggaa ttcatgctt gatgacgctt 120  
ctgtatctat attcatcatt ggaaacacca atgatatttt cttaatgtt gcctggcata 180  
atccctggaaa actgataaca caatgaaatt cttccactgt gcttaatttt accctctgaa 240  
ttctccattt ctcccataat catcattaca actgaactct ggaaataaaa cccatcatta 300  
ttaactcatt atcaaatcac gct 323

<210> 103

<211> 165

<212> DNA

<213> Artificial Sequence

<220>

<223> PTM binding domain

<400> 103

gctagcaata atgacgaagc cgcccccac gctcaggatt cacttgcctc caattatcat 60  
cctaaggcaga agtgtatatt cttatttgta aagattctat taactcattt gattcaaaat 120  
atttaaaata cttccgtttt cacctactct gctatgcacc cgccg 165

<210> 104  
<211> 225  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> Trans-splicing domain of PTM

<400> 104  
aataatgacg aagccgcccc tcacgctcag gattcacttg ccctccaatt atcatcctaa 60  
gcagaagtgt atattcttat ttgtaaagat tcttattaact catttgcattt aaaaatattt 120  
aaatacttcc tgtttcacct actctgctat gcacccgcgg aacatttatta taacgttgct 180  
cgaatactaa ctggtacctc ttctttttt tttgataatcc tgtag 225

<210> 105  
<211> 3069  
<212> DNA  
<213> Artificial Sequence

<220>  
<223> CFTR PTM sequence

<400> 105  
acttcacttc taatgtatgat tatgggagaa ctggagcctt cagagggtaa aattaagcac 60  
agtgaagaa tttcattctg ttctcagttt tcctggattt tgcctggcac cattaaagaa 120  
aatatcatct ttgggtttc ctatgtgaa tatagataca gaagcgtcat caaagcatgc 180  
caactagaag aggacatctc caagtttgcg gagaagaca atatagttt tggagaaggt 240  
ggaatcacac tgagttggagg tcaacgagca agaatttctt tagcaagagc agtatacaaa 300  
gatgctgatt tgtattttt agacttcctt tttggataacc tagatgtttt aacagaaaaaa 360  
gaaatatttg aaagctgtgt ctgtaaactg atggctaaaca aaacttaggt tttggtcact 420  
tctaaaatgg aacatttaaa gaaagctgac aaaatattaa ttttgcattt agtagcagc 480  
tattttatg ggacattttc agaactccaa aatctacagc cagactttt tagcaacttc 540  
atggatgtg attcttcga ccaatttagt gcagaaagaa gaaattcaat ccttaactgag 600  
acttacacc gtttctcatt agaaggagat gtcctgtct cctggacaga aacaaaaaaa 660  
caatctttt aacagactgg agagtttggg gaaaaaaagga agaattctat tctcaatcca 720  
atcaactcta tacgaaaattt ttccattgtg caaaagactc ccttacaaat gaatggcatc 780  
gaagaggatt ctgatgagcc tttagagaga aggctgtctt tagtaccaga ttctgagcag 840  
ggagaggcga tactgcctcg catcagcgtg atcagcaactg gccccacgct tcaggcacga 900  
aggagggcgt ctgtcctgaa cctgatgaca cactcagttt accaagggtca gaacattcac 960  
cgaaagacaa cagcatccac acgaaaagtg tcactggccc ctcaggcaaa cttgactgaa 1020  
ctggatataat attcaagaag gttatctcaa gaaactggct tggaaataag tgaagaaatt 1080  
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gtgattatca ccagcaccag ttctgttattt gtgtttaca tttacgtggg agtagccgac 1380  
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tcgaaaattt tacaccacaa aatgttacat tctgttcttc aagcacctat gtcaaccctc 1500  
aacacgttga aagcaggtgg gattcttaat agattctcca aagatatacg aattttggat 1560

gaccttctgc ctcttaccat atttgacttc atccagttgt tattaattgt gattggagct 1620  
atagcagttg tcgcagttt acaaccctac atctttgttgc caacagtgc agtgatagtgc 1680  
gctttatta tggtgagagc atatttctc caaacctcac agcaactcaa acaactggaa 1740  
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ggagaaggaa gagttgttat tattcactgtt ttagccatga atatcatgag tacattgcag 2040  
tgggctgtaa actccagcat agatgtggat agcttgatgc gatctgttag ccgagtcttt 2100  
aagttcatttgc acatgccaac agaaggtaaa cttaccaagt caaccaaacc atacaagaat 2160  
ggccaaactctcgaa cgaaagttat gattatttgag aattcacacg tgaagaaaaga tgacatctgg 2220  
ccctcagggg gccaaatgac tgtcaaagat ctcacagcaa aatacacaga aggtggaaat 2280  
gccatatttag agaacatttc cttctcaata agtccttgcc agagggtggg cctttggga 2340  
agaactggat caggaaagag tactttgtta ttagctttt ttagactact gaacactgaa 2400  
ggagaaatcc agatcgatgg tgggtcttgg gattcaataa ctttgcacaca gtggagggaaa 2460  
gccttggag tgataccaca gaaagtattt attttttctg gaacatttag aaaaaacttg 2520  
gatccctatg aacagtggag tgatcaagaa atatggaaat ttgcagatga gttgggctc 2580  
agatctgtga tagaacagtt tcctggaaag cttgactttg tccttgcgttga tggggctgt 2640  
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agaagaactc taaaacaagc atttgcgtat tgacacagtaa ttcttgcgttga acacaggata 2820  
gaagcaatgc tggaaatgcca acaatttttgc gtcatagaag agaacaaagt gcggcagttac 2880  
gattccatcc agaaactgtt gAACGAGAGG AGCCTCTCC GGCAAGCCAT CAGCCCTCC 2940  
gacagggtga agctcttcc ccaccggaac tcaagcaagt gcaagtctaa gccccagatt 3000  
gctgctctga aagaggagac agaagaagag gtgcaagata caaggcttca tcattcatcat 3060  
catcattag 3069

<210> 106

<211> 131

<212> DNA

<213> Artificial Sequence

<220>

<223> Binding domain of mouse factor VIII PTM

<400> 106

ctcgagctta cctgaactaa ttttttagaa tattaaatc ctaagctttt atatctctat 60

ccctctatct tttgctctct atccaatttt tattaactta gactttaaaa agaaacttat 120

gagaaaaatt t 131

<210> 107

<211> 71

<212> DNA

<213> Artificial Sequence

<220>

<223> Spacer sequence of PTM

<400> 107

ccgcggaca ttattataac gttgtcgaa tactaactgg tacctttctt ttttttttg 60

atatcctgca g

71

<210> 108

<211> 527

<212> DNA

<213> Artificial Sequence

<220>

<223> Chicken beta actin promoter sequences

<400> 108

ccatggtcga cgtagcccc acgttctgct tcactctccc catctcccc ccctccccac 60  
cccccaattt gtatttattt atttttaat tattttgtgc agcgatgggg gcgggggggg 120  
ggggggggcg cgccgcaggc ggggcggggc ggggcgaggg gcggggcggg gcgaggcgga 180  
gaggtgcggc ggcagccaat cagagcggcg cgctccgaaa gttcctttt tcgcgaggcg 240  
gcggcggcgg cggccctata aaaagcgaag cgcgcggcg ccgggagtcg ctgcgacgct 300  
gccttcgccc cgtgccaacc tccgcctcga gcttacctga actaatttt tagaatatta 360  
aaatcctaag cttttatact cctatccctc tatctttgc tctctatcca attttatta 420  
acttagactt taaaaagaaa cttatgagaa aaattccgc ggaacattat tataacgtt 480  
ctcgaatact aactggtacc tcttctttt ttttgatat cctgcag 527

<210> 109

<211> 169

<212> DNA

<213> Artificial Sequence

<220>

<223> Sequence not included in construct

<400> 109

cgccgcctcg cgccgccccgc cccggctctg actgaccgcg ttactccac aggtgagcgg 60  
gcgggacggc ctttcctc cgggctgtaa ttagcgctt gttaatcac ggcttgtttc 120  
ttttctgtgg ctgcgtaaa gccttgaggg gctccggag gaattcgta 169

<210> 110

<211> 42

<212> DNA

<213> Artificial Sequence

<220>

<223> F8 PTM sequences

<400> 110

ggagtcgctg cgacgctgcc ttccggccgt gccaacctcc gc

42

<210> 111

<211> 22

<212> DNA

<213> Artificial Sequence

<220>

<223> F8 PTM sequences

<400> 111

ctcgagcacc gatatcgtaa ct

22

<210> 112

<211> 53

<212> DNA

<213> Artificial Sequence

<220>

<223> Exon 26, Flag tag, stop sequences of mouse factor

VIII PTM

<400> 112

gaggccccagc agcaatacga ctacaaggac gacgatgaca agtgagttt aac

53

<210> 113

<211> 71

<212> DNA

<213> Artificial Sequence

<220>

<223> Spacer sequences of human or canine factor VIII

PTM

<400> 113

cccgcgaaaca ttattataac gttgctcgaa tactaactgg taccttcttct tttttttttt 60  
atatctcqca g 71